REMARKS

There have been no amendments made to the specification, claims, or drawings. Applicant notes that claims 20-23 were previously cancelled without prejudice. Upon entry of this amendment, claims 1-19 and 24 will be pending in the application. The Applicant further notes that claims 1-8 and 15-20 were previously withdrawn, under traverse, from consideration based on a restriction of invention so that claims 9-14 and 24 are presently under consideration.

Request for clarification of claims under consideration.

Applicant notes that the Office communication mailed on 5/18/2004 appears to contain an error in the Disposition of Claims section. In particular, line 4a indicates that claims 10-19 are withdrawn and line 6 indicates that claims 1-9 and 24 are rejected. In addition, in the Election/Restrictions section of the Detailed Action, the Examiner states that "[c]laims 10-19 remain withdrawn from consideration as being drawn to a nonelected invention". Furthermore, in the substantive portion of the Office Action the Examiner proceeds to discuss claim 9-14, and 24.

Applicant assumes that the Examiner has misstated the status of the claims. Applicant respectfully directs the Examiner's attention to the January 20, 2004 Response wherein it was noted that claims 1-8 and 15-20 had been withdrawn, under traverse, from consideration based on a restriction of invention. Claims 9-14 and 24 remain under consideration in this application. Applicant respectfully requests that the Examiner clarify and correctly state the status of the claims in the next Office correspondence.

Claim Rejections Under 35 U.S.C. §112 First Paragraph.

Claims 9-14 and 24 have been rejected under 35 U.S.C. §112, first paragraph, as failing to comply with the enablement requirement.

- **The legal burden necessary to properly assert a 35 U.S.C. §112, first paragraph rejection.**

35 U.S.C. section 112, first paragraph reads:

> The specification shall contain a written description of the invention and of the manner and process of making and using it, in such full, clear, concise, and exact terms as to enable any person skilled in the art to which it pertains, or with which it is most nearly connected, to make and use the same, and shall set forth the best way contemplated by the inventor of carrying out his invention.

Additionally, the courts have interpreted the enablement requirement to require that the specification teach those in the art to make and use the invention without "undue experimentation". As set out in In re Wands, 858 F.2d 731, 737; 8 USPQ2d 1400, 1404 (Fed. Cir. 1988), factors to be considered in determining whether required experimentation is undue include:

1. The breadth of the claims;
2. The nature of the invention;
3. The state of the prior art;
4. The level of a person of ordinary skill;
5. The level of predictability in the art;
6. The amount of direction provided by the inventor;
7. The existence of working examples in the specification; and
8. The quantity of experimentation needed to make or use the invention based on the content of the disclosure.

The courts have pointed out that "[n]ot every last detail [of an invention need] be described [in a patent specification], else patent specifications would turn into production specifications, which they were never intended to be." In re Gay, 135 USPQ 311,316 (C.C.P.A. 1962). Citing the opinion in Gay, the Board of Patent Appeals and Interferences echoed this point in its statement that " the law does not require a specification to be a blueprint to satisfy the requirement for enablement under 35 U.S.C. 112, first paragraph," Staehelin v. Secher, 24 USPQ2d 1513, 1516 (Bd. Pat. App. & Int. 1992). Even more broadly, the MPEP states the specification need not disclose what is well known to those skilled in the art and preferably omits that which is well known to those skilled and already available to the public. See MPEP section 2164.05(a).

The United States Patent and Trademark Office recognizing the above legal

authority has promulgated Training Materials For Examining Patent Applications With Respect To 35 U.S.C. 112, First Paragraph-Enablement Chemical/Biotechnical Applications. As stated in these training materials at section III, paragraph 6, with bolding added: "It is improper to conclude that a disclosure is not enabling based on an analysis of only one of the above [Wands] factors while ignoring one or more of the others. The examiner's analysis **must** consider all the evidence related to each of these factors, and any conclusion of non-enablement **must** be based on the evidence as a whole."

- **The Office communication assertions concerning enablement.**

The Office Communication asserted on page 3 that:

> The claims are not enabled for treating Alzheimer's, Parkinson's or lysosomal disorders". There is no known cure for treating Alzheimer's or Parkinson's thus treating is also not known. No known treatment can keep the disorders from happening. In fact, many people in the United States alone have such ailments and never get better. The evidence on record does not show that one who has such ailments ever gets any better, so how can one claim such treatments when they do not do what they claim they do? Without scientific data showing the diseases are prevented or cured then the treatment of diseases is just unknown since patients with such diseases never get any better.
>
> As far as "lysosomal storage disorders" go, they are also not enabled and cannot be determined as to what they would do. Applicant has claimed two specific disorders (Alzheimer's and Parkinson's) but it is not clear what would encompass "lysosomal storage disorders". Without more the claims are simply not enabled by the specification.

This is apparently the entirety of the Office communication rejection under 35 U.S.C. §112, first paragraph.

- **The Office communication assertions do not meet the required legal burden to assert a 35 U.S.C. §112, first paragraph rejection.**

As discussed above the United States Patent and Trademark Office has stated that: "It is improper to conclude that a disclosure is not enabling based on an analysis of

only one of the above [Wands] factors while ignoring one or more of the others. The examiner's analysis **must** consider all the evidence related to each of these factors . . ."

- The Office communication NEVER considers or discusses ANY of the Wands factors at all.
- The Office communication NEVER considers or discusses how the evidence relates to ANY of the Wands factors.

Applicant respectfully traverses the above rejection under 35 U.S.C. §112, first paragraph and asserts that it should be explicitly withdrawn. If the Examiner maintains a rejection under 35 U.S.C. §112, first paragraph than the Examiner should include an analysis applying ALL of the Wands factors to Applicant's specification and the art in this area. Claims 9-14 and 24 are patentable for at least this reason.

- **The Office communication assertions are contrary to knowledge in the art.**

The Office communication rejection appears to center around the position that: "[t]here is no known cure for treating Alzheimer's or Parkinson's thus treating is also not known." More simply, the Office communication appears to assert that cure = treatment.

The Office position appears to be misplaced, incorrect and fundamentally lacking technical support. There are a number of diseases for which no cure is known but for which treatment is possible. For example, diabetes can not be cured, however diabetics can be treated with diet or insulin. As another example HIV can not be cured, however drugs are available to treat the disease. Thus, it is beyond argument that a disease can be treated even if that same disease can not be cured.

The Office position with regard to treatments for, at least, Alzheimer's is also misplaced, incorrect and fundamentally lacking technical support. This is evidenced by the plethora of known treatments for this disease. For example, the United States Food and Drug Agency (FDA) specifically stated, as early as 1992, that there are "**[s]everal compounds for the treatment of Alzheimer's disease are under development or**

testing. (emphasis added)" FDA's ALZHEIMER'S DISEASE UPDATE, T92-43, page 1, Sept. 24, 1992, (copy enclosed). Furthermore, on September 9, 1993 the Food and Drug Administration approved a drug "**specifically to treat symptoms of Alzheimer's disease** (emphasis added)" and distinguished the term "treatment" from the term "cure" in stating the approved drug was "not a cure for Alzheimer's disease". FDA Press Release, P93-37, September 9, 1993 (copy enclosed). It is therefore excruciatingly clear that at the time the Applicant filed the present application treatments for, at least, Alzheimer's were well known.

With regard to Parkinson's disease, Applicant directs the Examiner's attention to a published article[1] (copy enclosed) which clearly indicates that as early as, at least, 1998 there existed a "**primary treatment for PD (Parkinson's Disease)** (emphasis added)". Based on at least this showing the Examiner's asserted position with regard to the nonexistence of treatments for Parkinson's disease appears to be in clear error. Claims 9-14 and 24 are patentable for at least this reason.

- **Applicant's claims reciting Lysosomal Storage Disorder are enabled.**

The Examiner has stated that "[a]s far as "lysosomal storage disorders" go, they are not enabled and cannot be determined as to what they would do." Applicant respectfully reminds the Examiner that "[p]atent documents are written for persons familiar with the relevant field; the patentee is not required to include in the specification information readily understood by practitioners, lest every patent be required to be written as a comprehensive tutorial and treatise for the generalist, instead of a concise statement for persons in the field. Thus resolution of any ambiguity arising from the claims and specification may be aided by extrinsic evidence of usage and meaning of a term in the context of the invention." Verve, LLC v. Crane Cams, Inc., 311 F.3d 1116, 1119, 65 USPQ2d 1051 (Fed. Cir. 2002); See Bayer AG v. Schein Pharmaceuticals, Inc., 301 F.3d 1306, 1314, 64 USPQ2d 1001 (Fed. Cir. 2002) ("Because an enabling disclosure by definition turns upon the objective understanding of a skilled artisan, the

[1] Altered Thalamic Response to Levodopa in Parkinson's Patients With Dopa-induced Dyskinesias, Tamara Hershey et al. Proc. Natl. Acad. Sci. USA, Volume 95, pp 12016-12021, (September 1998)

enablement requirement can be met by reference to the knowledge of one of ordinary skill in the relevant art."); S3 Inc. v. nVIDIA Corp., 259 F.3d 1364, 1371, 59 USPQ2d 1745 (Fed. Cir. 2001) ("The law is clear that patent documents need not include subject matter that is known in the field of the invention and is in the prior art, for patents are written for persons experienced in the field of the invention).

The Applicant respectfully asserts that the application, as filed, clearly enables the claim since it is well known that lysosomal disruption is associated with, among other things, the intracellular build up of protein fragments and aggregates which in turn are associated with neurodegenerative disorders. Specification, page 1, paragraph 2. In support of this assertion and as discussed by the courts, "the enablement requirement can be met by reference to the knowledge of one of ordinary skill in the relevant art". Bayer AG, at 1314 (Fed. Cir. 2002). Applicant has therefore enclosed with this paper several publications[2] which clearly identify that the phrase "lysosomal storage disorder" was well known in the art at the time the application was filed. Applicant notes that one reference, published in 1998, states that lysosomal storage disorders had been known for at least "a quarter of a century"[3] This reference specifically discusses the effect lysosomal storage disorders have on the intracellular accumulation of metabolic products. One skilled in the relevant art would understand what was encompassed by the phrase "lysosomal storage disorder" and would find the Applicant's specification to provide adequate guidance on how to use the claimed compositions. For example, the Applicant directs the Examiner's attention to at least Example 8 on page 19 of the specification. As such, Applicant respectfully requests that the rejection of the claims based on the language "lysosomal storage disorders" be withdrawn. Claims 9-14 and 24 are patentable for at least this reason.

---

[2] Gene Therapy of Lysosomal Storage Disorders, A. Salvetti, J. M. Heard, O. Danos, British Medical Bulletin, Vol. 51, No. 1, 106-122 (1995); Prevalence of Lysosomal Storage Disorders, Peter J. Meikle et al. , Jama, Vol. 281, No.3, 249-254 (1999); Cellular Pathology of Lysosomal Storage Disorders, Sidney Weisner, Rose F. Kennedy, Brain Pathology, vol. 8, 175-193 (1998). See also citation and abstracts of 48 items obtained from a search of PubMed for publications prior to the application's filing date having "lysosomal storage disorders" in the title.

[3] Cellular Pathology of Lysosomal Storage Disorders at page 175.

- **The enablement of Applicant's claims is supported by more recent scientific investigation.**

In one embodiment Applicant's invention enhances lysosomal function using a lysosomal modulating compound. Enclosed herewith is the cover page from an article published by the Journal of Neuroscience in 2004[4]. The abstract of this article states that: ". . . enhancing lysosomal function may be a potential therapeutic strategy to halt or even prevent the pathogenesis of Parkinson's disease and other Lewy body diseases." Thus, there is scientific evidence published in medical journals supporting the enablement of Applicant's claims. Claims 9-14 and 24 are patentable for at least this reason.

- **Applicant respectfully requests that the Examiner produce a Personal Knowledge Affidavit or Declaration.**

The Examiner, as discussed above, has based the rejection of claims 9-14 and 24 on the factual assertion that "treating (Alzheimer's or Parkinson's) is also unknown". Applicant has submitted copies of statements from the FDA showing the Examiner's statement to be in clear error. However, the Applicant assumes that the Examiner must have been aware of, at the time the May 18, 2004 Office Action was mailed, the Administrative Procedure Act (APA) which required that the Examiner apply a "substantial evidence" standard of review when relying on "common knowledge in the art or well known prior" See MPEP 2144.03. As such, the Examiner's statement must be backed by adequate evidence, which supports a finding that treatment of Alzheimer's is unknown. Since the Examiner has not provided the Applicant with documentary evidence, the Applicant assumes that the rejection must be based on the Examiner's personal knowledge. As discussed in the MPEP at §2144.03 (C) the Examiner should "provide an affidavit or declaration setting forth specific factual statements and explanation to support" his finding that treatment for Alzheimer's is unknown.

---

[4] Clearance of alpa-Synuclein Oliugomeric Intermediates via the Lysosomal Degradation Pathway, He-Jin Lee, Farnaz Khoshaghideh, Smita Patel and Seung-Jae Lee, The Journal of Neuroscience, 24(8):1888-1896 (February 25, 2004).

- **Applicant Requests Withdrawal of the Rejection if Examiner Fails to Provide Substantial Evidence.**

If the Examiner is unable to provide the required affidavit or declaration supporting a finding that a treatment of Alzheimer's is unknown, Applicant respectfully requests that the rejection of claims 9-14 and 24 be explicitly withdrawn. Applicant's basis for this request rests on, at least, the grounds that the examination has failed to comport with 37 C.F.R. §1.104 Nature of Examination, which reads in one pertinent portion:

> (a) Examiner's action. (1) On taking up an application for examination or a patent in a reexamination proceeding, the Examiner shall make a thorough study thereof and shall make a thorough investigation of the available prior art relating to the subject matter of the claimed invention. The examination shall be complete with respect ...to the patentability of the invention as claimed (emphasis added), as well as with respect to matters of form, unless otherwise indicated.

Claim Rejections Under 35 U.S.C. §112 Second Paragraph.

Claims 9-14 have been rejected under 35 U.S.C. §112, second paragraph, as being indefinite or failing to particularly point and distinctly claim the subject matter which the Applicant regards as the invention. Specifically, the Examiner has stated that [I]t is not clear what is meant by the term, "lysosomal storage disorders".

Applicant notes that "the test for definiteness is whether one skilled in the art would understand the bounds of the claim when read in light of the specification" Miles Laboratory, Inc. v. Shandon Inc., 997 F.2d 870 (Fed. Cir. 1993), cert. denied, 510 U.S. 1100 (1994). It is clear, as previously discussed, what the bounds of the claim language "lysosomal storage disorders" encompass when read in light of the specification. Clearly one skilled in the relevant art would understand the term "lysosomal storage disorders" and bounds of the claims when read in light of the specification as evidenced by references such as: Gene Therapy of Lysosomal Storage Disorders, A. Salvetti, J. M. Heard, O. Danos, British Medical Bulletin, Vol. 51, No. 1, 106-122 (1995); Prevalence of Lysosomal Storage Disorders, Peter J. Meikle et al. , Jama, Vol. 281, No.3, 249-254 (1999); Cellular Pathology of Lysosomal Storage Disorders, Sidney Weisner, Rose F. Kennedy, Brain Pathology, vol. 8, 175-193 (1998).

Applicant also directs the Examiner's attention to the citation and abstracts of 48 items obtained from a search of PubMed for publications prior to the application's filing date having "lysosomal storage disorders" in the title. Applicant respectfully traverses this rejection and asserts that the rejection of claims 9-14 under 35 U.S.C. §112, second paragraph be withdrawn.

In summary, the Applicant has addressed each of the rejections within the present Office Action. It is believed the application now stands in condition for allowance, and prompt favorable action thereon is respectfully solicited.

Respectfully submitted,

Ben A. Bahr

By: 

Date: August 17, 2004

James E. Piotrowski
Registration No. 43,860
Alix, Yale & Ristas, LLP
Attorney for Applicants

750 Main Street- Suite 1400
Hartford, CT 06103-2721
(860) 527-9211

\\Patlaw5\C-PATLAW5\AYR saved docs\Filing Docs\Uconba\uconba 186 us\8-04 response.doc